

Restoration of memory in Alzheimer's disease: a new paradigm using neural stem cell therapy

Grant Award Details

Restoration of memory in Alzheimer's disease: a new paradigm using neural stem cell therapy

Grant Type: Disease Team Therapy Development - Research

Grant Number: DR2A-05416

Project Objective: To develop a cellular therapeutic for patients with Alzheimer's disease and to file an IND for a Phase 1, first-in-human trial. Project scope includes manufacturing, IND-enabling preclinical studies and the submission of an IND. Cell source is fetal-derived hNSC that will be delivered intraparenchymally into the hippocampus.

Investigator:

Name: Alexandra Capela

Institution: StemCells, Inc.

Type: PI

Name: Frank LaFerla

Institution: University of California, Irvine

Type: Co-PI

Disease Focus: Alzheimer's Disease, Neurological Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: \$8,901,641

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 2

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Grant Application Details

Application Title: Restoration of memory in Alzheimer's disease: a new paradigm using neural stem cell therapy

Public Abstract: Alzheimer's disease (AD), the leading cause of dementia, results in profound loss of memory and cognitive function, and ultimately death. In the US, someone develops AD every 69 seconds and there are over 5 million individuals suffering from AD, including approximately 600,000 Californians. Current treatments do not alter the disease course. The absence of effective therapies coupled with the sheer number of affected patients renders AD a medical disorder of unprecedented need and a public health concern of significant magnitude. In 2010, the global economic impact of dementias was estimated at \$604 billion, a figure far beyond the costs of cancer or heart disease. These numbers do not reflect the devastating social and emotional tolls that AD inflicts upon patients and their families. Efforts to discover novel and effective treatments for AD are ongoing, but unfortunately, the number of active clinical studies is low and many traditional approaches have failed in clinical testing. An urgent need to develop novel and innovative approaches to treat AD is clear.

We propose to evaluate the use of human neural stem cells as a potential innovative therapy for AD. AD results in neuronal death and loss of connections between surviving neurons. The hippocampus, the part of the brain responsible for learning and memory, is particularly affected in AD, and is thought to underlie the memory problems AD patients encounter. Evidence from animal studies shows that transplanting human neural stem cells into the hippocampus improves memory, possibly by providing growth factors that protect neurons from degeneration. Translating this approach to humans could markedly restore memory and thus, quality of life for patients.

The Disease Team has successfully initiated three clinical trials involving transplantation of human neural stem cells for neurological disorders. These trials have established that the cells proposed for this therapeutic approach are safe for transplantation into humans. The researchers in this Disease Team have shown that AD mice show a dramatic improvement in memory skills following both murine and human stem cell transplantation. With proof-of-concept established in these studies, the Disease Team intends to conduct the animal studies necessary to seek authorization by the FDA to start testing this therapeutic approach in human patients.

This project will be conducted as a partnership between a biotechnology company with unique experience in clinical trials involving neural stem cell transplantation and a leading California-based academic laboratory specializing in AD research. The Disease Team also includes expert clinicians and scientists throughout California that will help guide the research project to clinical trials. The combination of all these resources will accelerate the research, and lead to a successful FDA submission to permit human testing of a novel approach for the treatment of AD; one that could enhance memory and save lives.

Statement of Benefit to California:

The number of AD patients in the US has surpassed 5.4 million, and the incidence may triple by 2050. Roughly 1 out of every 10 patients with AD, over 550,000, is a California resident, and alarmingly, because of the large number of baby-boomers that reside in this state, the incidence is expected to more than double by 2025. Besides the personal impact of the diagnosis on the patient, the rising incidence of disease, both in the US and California, imperils the federal and state economy.

The dementia induced by AD disconnects patients from their loved ones and communities by eroding memory and cognitive function. Patients gradually lose their ability to drive, work, cook, and carry out simple, everyday tasks, ultimately losing all independence. The quality of life for AD patients is hugely diminished and the burden on their families and caregivers is extremely costly to the state of California. Annual health care costs are estimated to exceed \$172 billion, not including the additional costs resulting from the loss of income and physical and emotional stress experienced by caregivers of Alzheimer's patients. Given that California is the most populous state and the state with the highest number of baby-boomers, AD's impact on California families and state finances is proportionally high and will only increase as the AD prevalence rises.

Currently, there is no cure for AD and no means of prevention. Most approved therapies address only symptomatic aspects of AD and no disease-modifying approaches are currently available. By enacting Proposition 71, California voters acknowledged and supported the need to investigate the potential of novel stem cell-based therapies to treat diseases with a significant unmet medical need such as AD.

In a disease like AD, any therapy that exerts even a modest impact on the patient's ability to carry out daily activities will have an exponential positive effect not only for the patients but also for their families, caregivers, and the entire health care system. We propose to evaluate the hypothesis that neural stem cell transplantation will delay the progression of AD by slowing or stabilizing loss of memory and related cognitive skills. A single, one-time intervention may be sufficient to delay progression of neuronal degeneration and preserve functional levels of memory and cognition; an approach that offers considerable cost-efficiency.

The potential economic impact of this type of therapeutic research in California could be significant, and well worth the investment of this disease team proposal. Such an approach would not only reduce the high cost of care and improve the quality of life for patients, it would also make California an international leader in a pioneering approach to AD, yielding significant downstream economic benefits for the state.

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